

hemophilic joint health (0.67 vs. 0.73, $p < .001$) or disability (0.65 vs. 0.70, $p < .001$) as similar as in the results of Haemo-QoL. With patients who were either on a job or students ($n=467$, 77.2%), LPT was estimated at 127.81 hours per month on average. Of 467, patients with inhibitor or disability showed higher LPT compared to those without inhibitor (130.61 vs. 126.61 hours per month, $p=0.486$) or disability. (132.27 vs. 124.95 hours per month $p=.087$). **CONCLUSIONS:** The study findings suggest that patients' clinical characteristics should take into account for the management of hemophilia given patient-reported outcomes differed by clinical manifestations.

PSY92

BURDEN OF LUPUS NEPHRITIS (LN) AMONG PATIENTS MANAGED IN ROUTINE CLINICAL PRACTICES IN EUROPE (EU)

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OBJECTIVES: To assess the burden of LN in comparison to SLE patients without Nephritis (Non-LN) in routine clinical practices in EU. **METHODS:** A multi-center medical chart-review of adult (16–89yrs) SLE patients was conducted among rheumatologists and internal medicine physicians in UK/France/Germany/Italy/Spain (5EU). Physicians were recruited from a geographically representative sample in each country. Approx. 5 consecutive eligible persistent active or relapse remitting SLE patients currently managed as part of usual care were identified. Physicians abstracted de-identified patient data on disease characteristics, lab values and treatment patterns. LN and Non-LN cohorts were compared using descriptive statistics. **RESULTS:** 168 LN patients and 569 non-LN patients with SLE were analyzed. Patient characteristics included (LN/Non-LN): age (yrs): 40.2/42.7; % Female: 82.7/79.4; % Caucasian: 82.7%/88.8%; % full-time employment: 32.1/36.0; % part-time employment: 19.0%/22.0%. Among LN/Non-LN cohorts, frequency of clinic visits was: 9.5wks/10.6wks, % currently receiving care in in-patient setting was: 14.9/8.8, % hospitalized ≥ 1 in the past-year was: 49.4/29.7; mean # of organ manifestations was: 3.9/3.0. Top-5 organ manifestations were (% LN/Non-LN): musculoskeletal: 85.1/90.8, mucocutaneous: 85.1/84.8, haematologic: 51.8/52.2, renal: 100.0/5.9, pulmonary: 16.7/16.2; % experiencing a flare was (LN/Non-LN): 23.2/16.6. Renal biopsy was performed in 87.5% (LN) and 4.0% (Non-LN) of patients. Steroids were used by 82.1% (LN) and 69.4% (Non-LN). % patients with low C3 and C4 was LN: 66.3/48.1/Non-LN: 60.2/50.0; mean ESR scores were 40.2 (LN) and 36.8 (Non-LN). Among patients with available data, SELENA-SLEDAI scores were 11.5 (LN) and 8.5 (Non-LN). Humanistic burden (reported via physician ratings, on a scale of 0 (most impact) to 7 (least impact)) was (LN/Non-LN, mean scores): ability to perform every-day tasks: 5.0/5.3, ability to interact fully with family and friends: 5.4/5.6, and ability to work/keep employment: 4.5/5.0. **CONCLUSIONS:** LN cohorts had higher clinical and humanistic burden in 5EU in comparison to their non-LN SLE counterparts. Factors influencing the observed burden, including the therapeutic strategies used in these geographies warrant further investigation to manage SLE, and LN in particular, optimally.

PSY93

QUALITY OF LIFE (QOL) WITH PSORIASIS: ETHNOGRAPHY STUDY EVALUATING THE IMPACT OF PSORIASIS ON MODERATE TO SEVERE PATIENTS IN EUROPE (EU), FROM A PATIENT'S PERSPECTIVE

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OBJECTIVES: To qualitatively assess the impact of Psoriasis on patient QoL. **METHODS:** An ethnographic study with moderate/severe Psoriasis patients was conducted in 4EU (UK/France/Spain/Italy) and the US to explore patients' views on treatment options and Psoriasis impact on QoL. Anthropologists and ethnographers spent several-hours with consented patients and filmed their behaviours inside and outside their homes in everyday situations. 175 hours of recordings/notes were analysed to identify QoL-related themes: self-image, psychological effects of psoriasis (e. g., anxiety/depression), life-style changes due to Psoriasis, relationship with family, friends and colleagues. **RESULTS:** Study included 35 adult patients (4EU: 20, US: 15; mean age: 39 yrs; female: 65%). Patients described their appearance with a sense of disgust and self-loathing. Feelings of frustration were often expressed due to a perceived lack of control of their lives. Prior to biologic initiation, daily rituals absorbed good part of their day, including waking up earlier to apply creams, going to work earlier to check their appearance and cover patches of dead skin. Due to lack of cultural discourse and patient's difficulty in articulating Psoriasis' impact, partners and family did not know how to react nor did they realize the full extent of the problem. Difficulty in getting appropriate psychological support needed left them with a feeling of resignation. As a result, majority dealt with their issues in isolation. Most patients experienced social discriminations due to Psoriasis which led some to lie about their disease, keeping it a secret. Biologic experienced patients noticed a significant improvement physically, but psychological scarring remained. Despite their regained confidence owing to the effectiveness of biologics, they still did not discuss their condition with family and friends. **CONCLUSIONS:** Patients with Psoriasis experienced significantly lower quality of life and high psychological scarring. Ethnographic study design vividly depicted the unarticulated and emotional impact of Psoriasis on patients' everyday lives.

PSY94

HEALTH RELATED QUALITY OF LIFE OF PATIENTS AND THEIR CAREGIVERS IN RARE DISEASES RESULTS OF THE BURQOL-RD PROJECT IN HUNGARY

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OBJECTIVES: The Social Economic Burden and Health-Related Quality of Life in Patients with Rare Diseases in Europe (BURQOL-RD, <http://www.burqol-rd.com/>) project aimed to investigate disease burden and self-perceived health outcomes of patients and their caregivers in rare diseases in eight EU countries (Bulgaria, France, Germany, Hungary, Italy, Sweden, Spain, UK). **METHODS:** An online questionnaire

survey was developed for patients (adults/children) and caregivers. Patients were recruited by patient organisations in cystic fibrosis (CF), Prader-Willi syndrome (PWS), haemophilia (HEMO), Duchenne muscular dystrophy (DMD), epidermolysis bullosa (EB), fragile X syndrome (FXS), scleroderma (SCL), mucopolysaccharidosis (MPS), juvenile idiopathic arthritis (JIA) and histiocytosis (HIS). Demography and main characteristics were recorded. Patients' and caregivers' health state was assessed by the EQ-5D-5L, disability and caregivers' burden by the Barthel Index and Zarit Burden Interview questionnaires. **RESULTS:** In Hungary, 296 Hungarian patients (children: 161, 54%) participated in the study (CF 110, PWS 5, HEMO 58, DMD 57, EB 6, FXS 12, SCL 38 and MPS 10 patients), no data were obtained in JIA and HIS. Mean age among adults/children was 37.0 (SD 16.1) / 9.6 (SD=4.5) years, and disease duration was 18.5 (SD=14.4) / 7.1 (SD=4.5) years, respectively. The lowest average EQ-5D-5L score was found in MPS (0.134 / 0.070) and DMD (0.310 / 0.198) just alike with the Barthel Index (MPS: 35.6; DMD 58.0). Caregivers' (N=95) mean age was 39.2 (SD=8.0) years and their EQ-5D-5L score (0.797, SD=0.251) was not significantly different from the general populations' average. Satisfaction with health care (1–10 Likert scale) was the lowest in MPS, DMD and EB (mean 4.1, 5.2 and 4.3). **CONCLUSIONS:** Rare diseases induce substantial deterioration of patients' quality of life and impose burden on caregivers. Taking an integrated approach our results can serve for international comparisons and facilitate further investigations in other orphan diseases.

PSY95

IS THE DISEASE-SPECIFIC LUPUSQOL SENSITIVE TO CHANGES OF DISEASE ACTIVITY IN SYSTEMIC LUPUS ERYTHEMATOSUS PATIENTS AFTER TREATMENT OF A FLARE?

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OBJECTIVES: The aim of the UK multi-centre LupusQoL Sensitivity Study is to assess whether the LupusQoL, a systemic lupus erythematosus (SLE) specific, health-related quality of life measure is sensitive to change when disease activity improves or deteriorates. **METHODS:** Patients with SLE experiencing a flare (baseline) & requiring an increase in treatment were recruited. Assessments were undertaken at baseline & monthly for 9 months & included BILAG-2004 disease activity index & the LupusQoL with 8 domains and scores ranging from 0 (worst) to 100 (best HRQoL). LupusQoL domain scores when disease activity improved or deteriorated between consecutive time-points are reported as mean changes, with 95% CI constructed using robust standard errors to account for repeated patient assessments. **RESULTS:** Mean (SD) age was 40.9 (12.8) & duration since diagnosis was 9.3 (8.1) years for the 101 patients recruited; 94% females, 62.6% white Caucasians. At baseline all mean LupusQoL domain scores were <52. Scores for LupusQoL physical health (+4.0, 95%CI 1.9 to 6.1), pain (+7.7, 95%CI 4.8 to 10.5) & fatigue (+4.1, 95%CI 1.7 to 6.5) increased when BILAG improved. Scores for physical health (-4.9, 95% -9.4 to -0.4) and pain (-6.9, 95%CI -12.9 to -0.8) decreased with a major BILAG deterioration but changes with a minor deterioration were small and non-significant. The effects of improvements & deterioration in BILAG on the other LupusQoL domain scores were smaller. **CONCLUSIONS:** Improvement and deterioration of LupusQoL domain scores for physical health, pain & fatigue domain scores was seen in patients with significant changes in disease activity over 1 month. Sensitivity to change of other LupusQoL domains in relation to changes in disease activity may need to be evaluated over a longer interval as the more emotive type of response to the disease & its consequences may be latent and therefore not evident at monthly intervals.

PSY96

THE QUALITY OF LIFE OF PATIENTS TREATED WITH ROBOTIC VERSUS TRADITIONAL SURGERY RESULTS FROM AN ITALIAN OBSERVATIONAL MULTICENTER STUDY

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OBJECTIVES: To assess the Quality of Life (QoL), focusing on level of pain, of patients submitted to surgical interventions with robotic technique in comparison with traditional approach in the Italian setting. **METHODS:** The prospective multicentre study analysed the QoL of 699 patients submitted to surgical interventions, enrolled in 8 Italian Hospitals for the period February 2011-May 2014. The specialties were general, thoracic, gynecological surgery performed with open, manual laparoscopic or robotic technique. Patients completed two questionnaires: one related to pain intensity during the hospitalization and one fulfilled at home related to pain intensity and its impact on daily activities, mood, relationship, sleep. Pain was measured using the scale of facial expressions corresponding to the Visual Analog Scale. For each intervention and specialty, linear regression for repeated measure, corrected by length of stay and use of analgesics, were performed to explain level of pain during hospitalization and at home, respectively. **RESULTS:** Level of pain during hospitalization is significantly ($p=0.05$) lower in general and gynecological robotic surgery versus the open technique, but not versus laparoscopic, while robotic thoracic surgery presents significant differences with laparoscopic but not with open surgery ($p=0.059$). Level of pain at home is significantly different for robotic interventions versus open both in general and gynecological surgery; moreover, with respect to laparoscopic interventions, the differences are significant both in gynecological and in thoracic but not in general surgery. **CONCLUSIONS:** The study gives us insightful knowledge about QoL, focusing on pain, of patients submitted to surgical interventions with robotic technique vs traditional surgery. Further analysis are in progress to combine results on QoL, not only referred to level of pain, with clinical severity and other indicators of clinical efficacy as length of stay, operating

time, medical/surgical complications to refine a robust measure of effectiveness useful to perform cost effectiveness analysis.

PSY97

CHARACTERISTICS OF PATIENTS WITH SYSTEMIC LUPUS ERYTHEMATOSUS (SLE) CURRENTLY ON REMISSION, WITH ACTIVE DISEASE BUT NOT EXPERIENCING FLARE, AND THOSE EXPERIENCING FLARES IN CLINICAL PRACTICES IN EUROPE

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OBJECTIVES: To assess the characteristics of SLE patients currently on Remission (Group-1), currently with active disease but not experiencing a flare (Group-2) and those who are experiencing flares (Group-3) in Europe (EU). **METHODS:** A multi-center medical chart review of adult (16-89 yrs) SLE patients was conducted in 1Q2014 among rheumatologists/internal medicine physicians in UK/France/Germany/Italy/Spain (5EU). Physicians were recruited from a geographically representative sample in each country. Approx. 5 consecutive eligible persistent active or relapse remitting SLE patients currently managed as part of usual care were identified. Physicians abstracted de-identified patient data on disease characteristics, lab values and treatment patterns. Patient disease status and humanistic burden was assessed by physician per clinical judgment & patient interaction. **RESULTS:** 747 SLE patient charts were abstracted in 5EU (UK: 156/France: 149/Germany: 148/Italy: 146/Spain: 148); Group-1: 25.7% (range: 19.9% (Italy) –29.7% (Germany)), Group-2: 56.2% (range: 42.3% (UK) –66.4% (Italy)), Group-3: 18.1% (range: 13.5 (Germany) –28.8 (UK)). Patient characteristics included (Group-1/Group-2/Group-3): age (yrs): 41.4/43/40.6; % female: 81.8/80.2/77.8; % full-time employment: 38.0/34.5/32.6; % part-time employment: 25.5/20.5/17.8; % on sick leave: 3.6/7.6/17.0; % currently receiving treatment in in-patient setting: 3.1/7.6/28.1; % hospitalized >=1 in past-year: 23.4/32.1/55.6. Top-5 organ manifestations were (% Group-1/Group-2/Group-3): musculoskeletal: 90.6/89.5/88.1, mucocutaneous: 86.5/84.0/85.2, haematologic: 45.8/53.8/55.6, renal: 23.4/26.7/33.3, pulmonary: 15.1/13.6/26.7. In Group-1/Group-2/Group-3, % patients with low C3 and C4 were 33.3/53.9/73.4 and 31.6/53.2/77.5 and % anti-ds-DNA positive were 54.7/71.0/78.5. Humanistic burden (reported via physician ratings, on a scale of 0 (most impact) to 7 (least impact)) was (Group-1/Group-2/Group-3, mean scores): patient ability to perform every-day tasks: 6.11/5.17/4.37, patient ability to interact fully with family and friends: 6.20/5.45/4.90, and patient ability to work/keep employment: 5.76/4.80/3.87. **CONCLUSIONS:** Over half of the SLE patients had an active disease while one-in-five were experiencing a flare in this study cohort, with significant variations observed within 5EU. Clinical and humanistic burden varied based on patient disease status, with highest burden observed among those experiencing flares.

SYSTEMIC DISORDERS/CONDITIONS – Health Care Use & Policy Studies

PSY98

ORPHAN DRUG POLICY: APPROACHES TO MARKET ACCESS IN MULTIPLE COUNTRIES

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OBJECTIVES: Despite increasing policy guidance to encourage the development of and access to orphan drugs, the policy landscape indicates a lack of transparency and consistency across countries. The objective of this study is to compare the orphan drug policies of Japan, South Korea, and Taiwan to the policies in the EU. **METHODS:** A targeted literature review was conducted to identify papers pertaining to orphan drugs in Japan, South Korea and Taiwan, with a focus on quantitative analysis for policy-making related to pricing and reimbursement from the payer perspective. No limits were placed on language. Country specific policy websites were hand-searched. **RESULTS:** A total of 3465 abstracts were identified for screening. Of those, 104 were eligible for full-text screening. The definition of prevalence with regard to rare diseases in Japan, South Korea and Taiwan was stricter than in the EU. All of them had introduced regulations, guidelines and incentives to the development of orphan-designated drugs. Strict HTA requirements were waived for rare diseases, although cost-effectiveness data are referenced in South Korea. In Japan and Taiwan, prices were reduced for existing drugs with new orphan-designated indications. Within the existing benefits of national health insurance schemes or under the coverage of rare disease regulations, full reimbursement was given, which is different from the partial reimbursements observed in certain European countries. In some cases, the application of drug treatment can be approved prior to or without market authorisation. The availability of orphan drugs to patients was not inferior to that of the EU. **CONCLUSIONS:** While access in Japan, South Korea and Taiwan is seemingly no different to the EU, there are no consistent or transparent policies in place in these countries. Policy makers should prepare innovative schemes that offer warranties to both payers and patients and establish a systematic evaluation procedure for manufacturers.

PSY99

MCDA APPROACH TO RANKING RARE DISEASES IN RUSSIA: PRELIMINARY RESULTS

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OBJECTIVES: The ranking and prioritization of rare diseases are crucial in order to define for which of them state support measures are justifiable. For this purpose the set of 16 criteria to assess rare diseases (8 related to the characteristics of disease, and 8 to the characteristics of treatment) was proposed by experts, so multi-criteria decision (MCDA) analysis approach could be useful. The aim of the study was to assess the reliability of the criteria set and to determine the relative importance

of 16 criteria. **METHODS:** 85 experts were interviewed to estimate the importance of each criterion in the decision-making on financing MT for rare diseases. We used 10-point scale, where 10 points mean major importance to the priority indicator, and 1 point means minor importance. Mean estimates were calculated using descriptive statistics, then means were normalized. **RESULTS:** Respondents were 41 years on average (ranging from 23 to 64 years), and included 20 public servants, 16 health administrators, 32 practitioners, and 14 researchers. 44 respondents had a scientific degree. The most important criteria were characteristics of treatment - "Effect of treatment on quality of life" and "Effect of treatment on life expectancy" with 1 point each. The least important criteria were both characteristics of the disease - "Cognitive disorders as manifestations of the disease and "Additional burden on the daily lives of care-givers" with 0.28 and 0.1 respectively. **CONCLUSIONS:** Characteristics of treatment turned out to be more important for respondents than characteristics of disease, therefore characteristics of treatment should be given consideration when evaluating rare diseases to determine priority financing.

PSY100

PATTERNS AND TRENDS IN OPIOID USE IN IRAN FROM 2007 TO 2011

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OBJECTIVES: Opioid analgesics are proven to be safe and effective in malignant or nonmalignant pains. The consumption trend of six opioids (Morphine, Codeine, Oxycodone, Fentanyl, Pethidine, and Methadone) in Islamic Republic of Iran assumed as an indicator for prescription pattern and is evaluated during five year period (2007-2011) as the aim of this study. **METHODS:** The data of opioid analgesic consumption were collected from FDO (Food and Drug Organization) of Iran. The collected data were converted to DDD (Define Daily Dose) for each of six selected opioids in order to be compared accurately. **RESULTS:** Overall consumption of opioids was grown during the period of investigation. Putting six selected opioids into two groups – synthetic and non-synthetic -, the growth rate of synthetic ones are obviously higher than those of non-synthetics. Opioid analgesics consumption in 2011 was shown to be 4 times more than the opioid consumption in 2007. The CAGR (Compound Average Growth Rate) between 2007-2011 were reported 111.27%, 33.11%, 16.48%, 3.91%, 3.76%, and -41.63% for Oxycodone, Methadone, Fentanyl, Morphine, Pethidine and Codeine respectively. The growth rate of mentioned above opioids for the last year of investigation was reported 41.12%, 16.54%, 29.99%, -0.38%, 7.66%, 23.67% respectively. **CONCLUSIONS:** Like other low consuming countries, consumption of opioid analgesics in Iran is limited by means of different kinds of barriers. There are also parameters which set a specific orientation in opioid consumption. Analyzing this trend, results in defining the barriers and other parameters clearly.

PSY101

ORPHAN DRUG APPROVALS IN EUROPE: HISTORICAL REVIEW AND TRENDS

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OBJECTIVES: In Europe, orphan designation has been granted by European Medicines Agency since 2000. Molecules with orphan designation can benefit from a number of incentives to guarantee return on investment for manufacturers. Since introduction of orphan legislation, the number of Orphan Drugs (OD) has significantly increased. In 2012, total OD sales reached 13% of the whole pharmaceutical market. This study aims to analyse current situation and trends in OD approvals. **METHODS:** All ODs approved gaining marketing authorization was identified through EMA website. Extensions to a new disease were considered as an independent approval, while extensions of indication for the same condition were not considered. Information on drug status, indications, therapeutic area, and authorisation date was extracted. **RESULTS:** 77 orphan molecules were identified of which two have been withdrawn, and one orphan designation has expired. Yearly approvals continuously grow (from 2 ODs approved in 2002 to 12 and 8 drugs in 2012 and 2013 respectively). By mid-2014, already 6 were approved. There is a high imbalance within therapeutic area with 36% for oncology, 27% for endocrine, nutritional and metabolic disorders whereas the remaining 37% cover 11 therapeutic classes. No product has been approved for 7 therapeutic areas despite existence of rare disorders. Moreover, oncology is associated with the highest growth. **CONCLUSIONS:** Orphan drug market is rapidly growing but targeting selective therapeutic areas with high potential return on investment. The incentive policies happen to well function but discriminate among diseases. A review of orphan drug policies might be warranted to ensure patient equity in development of new OD.

PSY102

COMPARATIVE ANALYSIS OF HTA DECISIONS, PRICE AND REIMBURSEMENT LEVEL OF ORPHAN DRUGS IN FRANCE AND ITALY

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OBJECTIVES: While there exist a number of incentives to stimulate research and development of orphan drugs (OD), the Health Technology Assessment (HTA) agencies do not offer specific path for OD in most countries where it is left to payer value judgement. This leads to a high inequity in patient access to OD. The study aims at comparing the HTA decisions, price and reimbursement level of OD in France and Italy. **METHODS:** All OD assessed since 2000 were analysed. Prices, reimbursement rates and decision details were extracted for each drug using Farmadati Italia database for Italy and Transparency committee reports, AMELI's national health insurance and Thériaque databases for France. **RESULTS:** Among 74 OD approved in Europe 66 are available in Italy compared to 53 in France. All ODs available in France are officially available in Italy. The average delay between the market authorization and the price and reimbursement decision was about 16 and 17 months in France and Italy, respectively. In France all available drugs are 100% reimbursed through hospital, 36 molecules are available in retail pharmacy with reimbursement from